CIRM Awards More than $25 Million To Develop New Treatments for Disabling Disorders

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San Francisco, CA – With a goal of supporting projects that have the best chance of delivering effective therapies to patients in need CIRM, California's stem cell agency, has awarded $25.2 million to research into seven deadly or disabling disorders including Huntington's disease, Spina Bifida, and osteoarthritis.

The funding is part of the agency's Preclinical Development Awards program. This supports projects that use or target stem cells, have shown potential in early stage research and are now ready to move one step closer to clinical trials in people.

"This investment will let us further test the early promise shown by these projects," says Jonathan Thomas, Ph.D., J.D., Chair of the agency's governing Board. "Preclinical work is vital in examining the feasibility, potential effectiveness and safety of a therapy before we try it on people. These projects all showed compelling evidence that they could be tremendously beneficial to patients. We want to help them build on that earlier research and move the projects to the next level."

The successful programs target Spina Bifida, osteoarthritis, diabetic wounds, Alzheimer's disease, Huntington's disease, osteochondral defects, and Severe Combined Immunodeficiency (SCID), also known as 'bubble baby' disease.

Spina Bifida is a disabling condition where the baby's spinal cord fails to develop properly. In their proposal researchers at the University of California, Davis, want to take a stem cell therapy they have developed and use it to treat fetuses in the womb, effectively fixing the problem before it is a problem. They have already done much of the groundwork and in their application say the next step is to: "Optimize this stem cell product, validate its effectiveness, determine the optimal dose, and confirm its preliminary safety in order to translate this new treatment to clinical trials. Stem cell therapy for Spina Bifida could cure this devastating disease, alleviating a massive burden on children, families and society."

Here are the successful applications:

Application	Researcher	Institution	ICOC Committed funding
PC1-08103 Spina Bifida	Diana Farmer	U.C. Davis	$2,184,032
PC1-08142 Osteoarthritis	Peter Schultz	California Institute for Biomedical Research	$2,633,592
PC1-08118 Diabetic wounds	Rivkah Isseroff	U.C. Davis	$5,039,008
PC1-08117 Huntington's	Leslie Thomson	U.C. Irvine	$4,951,623
PC1-08111 SCID	Matthew Porteus	Stanford	$1,000,000

PC1-08086 Alzheimer's disease	David Schubert	Salk	$1,737,271
PC1-08128 Osteochondral defects	Darryl D'Lima	Scripps Health	$7,660,211

Three other projects were listed in Tier 2, which means that while they had scientific merit the independent review panel was not sufficiently impressed by at least one aspect of the application to recommend that they be funded.

CIRM President & CEO, C. Randal Mills, Ph.D., says the previous system that this application was filed under doesn't allow for changes to help applicants improve their submissions and re-file again in a timely manner. That's about to change.

"Our old system was too inflexible and didn't give our expert reviewers a chance to help the applicants improve their programs. Basically if a program wasn't funded it was finished. But, under CIRM 2.0 we are creating a program that allows projects that are considered promising, but flawed in some way, to refine their applications, resolve those flaws, and then resubmit within a short time. We are creating a system that has the flexibility needed to ensure we don't throw away promising science because of technical, correctable problems."

The researchers who fell short this time around will be able to resubmit their amended applications after the Board approves the CIRM 2.0 Translational program. That could happen as soon as July. Under the old system applicants would have to wait up to 22 months for approval. Under CIRM 2.0 that will happen in just 120 days.

The Board also approved an application for the Tools and Technology Award that had been deferred from the January meeting. The application – RT3-07678 – "A small molecule tool for reducing the malignant potential in reprogramming human iPSCs and ESCs" – was placed in Tier 2 by the independent GWG review panel. However the CIRM science team recommended it for funding saying it addresses a significant bottleneck in stem cell research. The vote means that Carla Koehler at UCLA will now get $1.34 million for that research.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and to act with a sense of urgency commensurate with that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $3 billion in funding and over 280 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of medicine closer to reality.

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